INSPIRE: A Phase 3 Open-Label, Multicenter Study to Evaluate the Safety and Tolerability of LIQ861 in Pulmonary Arterial Hypertension (PAH) – Exploratory Efficacy Endpoints Analysis at Month 2

Nicholas S. Hill,1 Jeremy P. Feldman,2 Sandeep Sahay,3 Deborah Levine,4 Robert F. Roscigno,5 Toby A. Vaughan,6 Todd M. Bull,7 on behalf of the INSPIRE Investigators

1Tufts University School of Medicine, Boston, MA; 2Arizona Pulmonary Specialists, Ltd., Phoenix, AZ; 3Houston Methodist Hospital, Houston, TX; 4University of Texas Health Science Center, San Antonio, TX; 5Liquidia Technologies, Morrisville, NC; 6University of Colorado School of Medicine, Aurora, CO

BACKGROUND

LIQ861 is a novel, oral, dry-powder formulation of imipronsol developed by Liquidia Technologies Inc. Preclinical studies in a Rhesus Multicenter study of PAH patients (LTI-301; safety outcomes); and a global, open-label, phase 3, multicenter study of PAH patients (LTI-317; safety outcomes). The INSPIRE study evaluated the safety and tolerability of LIQ861 in PAH patients who were on a stable dose of Tyvaso® for ≥3 months. This exploratory efficacy analysis of the INSPIRE study through Month 2 is reported.

METHODS

• The INSPIRE study was a phase 3, open-label, multicenter study of PAH patients (LTI-301; safety outcomes); and a global, open-label, phase 3, multicenter study of PAH patients (LTI-317; safety outcomes). The INSPIRE study through Month 2 is reported.

• METHODS

RESULTS

• LIQ861 was well tolerated with most adverse events (AEs) being mild or moderate in severity and classified as treatment-related for both cohorts.

• Transitions

- Adverse events were the most common reason for discontinuation in both groups, with no discontinuations due to AEs for the Add-On group.

- Overall, the majority of patients were female (n=99; 81.8%), white (n=96; 79.3%), and non-Hispanic (n=101; 83.3%), with an overall mean age of 54.2 years at Baseline (Table 1).

• Exploratory Endpoints

- Improvement in mPVR was observed with an overall median increase of 10.1% at Month 2 (Figure 3).

- A larger percentage of patients met 2 or 3 PAH low-risk criteria at Month 2 compared with Baseline.

- More than 70% of patients titrated to a LIQ861 dose ≥79.5 mcg, which is approximately equivalent to 54 mcg Tyvaso® per puff.

- Emirazolim questionnaire showed an improved total score (>5 point decrease) in addition to improvements in Kakio (≥50 m) and CIV (≥10 m).

- A larger percentage of patients met 2 or 3 PAH low-risk criteria at Month 2 compared with Baseline.

- A stable dose of Tyvaso® for ≥3 months (Transitions)

- An additional exploratory endpoint for Transitions patients only was patient-reported satisfaction with the RS00 Model 8 dry-powder inhaler device compared with the Tyvaso® Inhalation System.

- The majority of patients preferred the RS00 Model 8 dry-powder inhaler device compared with the Tyvaso® Inhalation System.

- The primary endpoint was the incidence of treatment-emergent adverse events and serious adverse events at Month 2.

- The percentage of patients who met 2 or 3 PAH low-risk criteria (based on European guideline-specified criteria) increased from Baseline to Month 2, with a larger shift in Add-On patients compared with Transitions (Figure 8).

- No clinically meaningful change was observed in NT-proBNP with an overall mean increase from Baseline to Month 2 of 6.6% (5.3%).

- At Week 2, the majority of patients in the Transitions group avg scored >60 for the RS00 Model 8 dry-powder inhaler (Table 7).

- The majority of Transitions patients preferred the RSI Model 8 dry-powder inhaler to the Tyvaso® Inhalation System

- The RS00 Model 8 dry-powder inhaler compared with the Tyvaso® Inhalation System.

- The MLWHF questionnaire showed an improved total score (>5 point decrease) in addition to improvements in Kakio (≥50 m) and CIV (≥10 m).

- The MUPI chart demonstrated an overall median increase of 4.5 points in addition to improvements in Emirazolim, Physical and Emotional Dimension scores.

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